

Title (en)  
COMPOSITIONS FOR DRG-SPECIFIC REDUCTION OF TRANSGENE EXPRESSION

Title (de)  
ZUSAMMENSETZUNGEN ZUR DRG-SPEZIFISCHEN REDUKTION DER TRANSGENEXPRESSION

Title (fr)  
COMPOSITIONS POUR LA RÉDUCTION SPÉCIFIQUE DE DRG DE L'EXPRESSION DE TRANSGÈNE

Publication  
**EP 4048785 A4 20240327 (EN)**

Application  
**EP 20880317 A 20201022**

Priority  
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• US 201962934915 P 20191113  
• US 2019067872 W 20191220  
• US 202062972404 P 20200210  
• US 202063005894 P 20200406  
• US 202063023602 P 20200512  
• US 202063038514 P 20200612  
• US 202063043600 P 20200624  
• US 2020056881 W 20201022

Abstract (en)  
[origin: WO2021081217A1] Provided herein are nucleic acid sequence encoding hIDUA and expression cassettes containing these coding sequences. Also provided are vectors, such as recombinant adeno-associated virus (rAAV) vectors having a vector genome including a hIDUA coding sequence operably linked regulatory sequences that direct expression of the hIDUA. Also provided are compositions containing these expression cassettes and rAAV vectors and methods of treating MPS1 or an associated syndrome such as Hurler, Hurler-Scheie and/or Scheie syndrome. The compositions and methods provided are further designed to selectively repress expression of hIDUA in dorsal root ganglia.

IPC 8 full level  
**C12N 9/24** (2006.01); **C12N 15/86** (2006.01)

CPC (source: EP IL KR US)  
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Citation (search report)  
• [I] WO 2019010335 A1 20190110 - UNIV PENNSYLVANIA [US]  
• [A] CHRISTIAN HINDERER ET AL: "Neonatal Systemic AAV Induces Tolerance to CNS Gene Therapy in MPS I Dogs and Nonhuman Primates", MOLECULAR THERAPY, vol. 23, no. 8, 1 August 2015 (2015-08-01), US, pages 1298 - 1307, XP055361153, ISSN: 1525-0016, DOI: 10.1038/mt.2015.99  
• [A] ERIKALLEN LYKKEN ET AL: "Recent progress and considerations for AAV gene therapies targeting the central nervous system", JOURNAL OF NEURODEVELOPMENTAL DISORDERS, BIOMED CENTRAL LTD, LONDON, UK, vol. 10, no. 1, 18 May 2018 (2018-05-18), pages 1 - 10, XP021256519, ISSN: 1866-1947, DOI: 10.1186/S11689-018-9234-0  
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• [A] CHRISTOPHER G. JANSON ET AL: "Comparison of Endovascular and Intraventricular Gene Therapy With Adeno-Associated Virus-[alpha]-L-Iduronidase for Hurler Disease", NEUROSURGERY, vol. 74, no. 1, 1 January 2014 (2014-01-01), US, pages 99 - 111, XP055324651, ISSN: 0148-396X, DOI: 10.1227/NEU.00000000000000157  
• See also references of WO 2021081217A1

Designated contracting state (EPC)  
AL AT BE BG CH CY CZ DE DK EE ES FI FR GB GR HR HU IE IS IT LI LT LU LV MC MK MT NL NO PL PT RO RS SE SI SK SM TR

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**US 2020056881 W 20201022**; AU 2020369570 A 20201022; CA 3155154 A 20201022; EP 20880317 A 20201022; IL 29237222 A 20220419; JP 2022524118 A 20201022; KR 20227016895 A 20201022; US 202017770137 A 20201022